April 23, 2015

Chairman Fred Upton
Representative Diana DeGette
House Energy & Commerce Committee
Washington, DC

Dear Chairman Upton & Representative DeGette,

The undersigned patient organizations wish to thank you and the Energy and Commerce Committee for the opportunity to be part of the 21st Century Cures Initiative development process. We applaud your efforts to truly engage the rare disease patient community through requests for white papers, hearings, roundtables, individual meetings and personal phone calls.

With more than 30 million Americans affected by rare diseases, the launch of the 21st Century Cures Initiative is our community's greatest hope for treatments. There has not been a greater opportunity to advance policy to improve drug development for rare diseases patients since the passage of the Orphan Drug Act more than 30 years ago.

We are pleased to see many provisions in the discussion draft that are supported by the rare disease community, including:

- Incorporating the patients perspective into the regulatory process with focus on risk vs benefit
- Empowering our nation's emerging scientists and researchers
- Creating economic incentives to encourage industry to develop drugs for unmet medical needs
- Advancing regulatory science to allow for the use of biomarkers and surrogate endpoints
- Enhancing the Food and Drug Administration's scientific capacity by improving access to adequate funding, recruiting world-class scientific and technical experts
- Funding for the National Institutes of Health

This first draft is an important first step and we look forward to working with you to ensure bipartisan legislation is introduced in both the House and the Senate in the coming months. Ninety five percent of the nearly 7,000 rare diseases have no approved treatments. Therefore, passage of bipartisan legislation is essential so that our families may see treatments developed during their lifetime.

Thank you for your continued leadership in this historic initiative. We are committed to work with you and advocate for bipartisan legislation that improves the lives of patients.

Sincerely,

EveryLife Foundation for Rare Diseases
Global Genes
Parent Project Muscular Dystrophy
Sarcoma Foundation of America
Little Miss Hannah Foundation

NGLY1.org
RASopathies Network USA
Saving Case & Friends - a Hunter Syndrome Research Foundation
The Mission Massimo Foundation for Leukodystrophies
Coalition Duchenne
Alternating Hemiplegia of Childhood Foundation
Noah's Hope
Batten Disease Support & Research Association
Relapsing Polychondritis Awareness & Support Foundation, Inc.
Klippel-Feil Syndrome Freedom
CADASIL Together We Have Hope
CureDuchenne
NTM Info & Research Inc.
Cure HHT
Mytonic Dystrophy Foundation
Rare & Undiagnosed Network
Pulmonary Fibrosis Advocates
GT23 Foundation
Bcureful
Lipodystrophy United
The AKU Society of North America
Organization for Rare Diseases in India
American Partnership for Eosinophilic Disorders (APFED)
Alstrom Syndrome International
cureCADASIL
Moebius Syndrome Foundation
Leiomyosarcoma Direct Research Foundation
National MPS Society
Gene Giraffe Project
The Foundation for Angelman Syndrome Therapeutics
I Have IIH Foundation
SCAD Alliance
The Association for Glycogen Storage Disease
Polycystic Kidney Disease (PKD) Foundation
Fabry Support & Information Group
International Pemphigus & Pemphigoid Foundation
Stiff Person Syndrome Action Network Inc NFP
BRBN Alliance
Phelan-McDermid Syndrome Foundation
Organic Acidemia Association
MLD Foundation
NBIA Disorders Association
BioPontis Alliance for Rare Diseases
Castleman's Awareness & Research Effort/Castleman Disease Collaborative Network
5p- Society
Dup15q Alliance
Foundation for Ichtyosis & Related Skin Types, Inc.
National Gaucher Foundation Inc.

Alliance For Cryoglobulinemia
Dysteratosis Congenita Outreach, Inc.
OsteoPETrosis Society